

Biomarin Pharmaceutical Inc Submission to the TGA Consultation on the Orphan Drugs Program Proposal – November 2016

Please see below the comments from Biomarin Pharmaceutical Inc regarding the TGA's proposal for revisions to the Australian Orphan Drugs program. The page references included in our comments refer to the TGA Consultation Document dated October 2016 (Version 1.1).

Page 15 – Modified Designation Process – Question 5

Comment 1:

The imposition of a time limit in which a sponsor must submit their registration application (i.e. 3-6 months from orphan designation) may create regulatory challenges for international organisations. Many international companies will target regulatory submission in Australia immediately following EU or USA regulatory approval however these processes are not always easy to predict with significant differences between “best case” and “worst case” scenario being commonly encountered. Consequently, it is a likely scenario that a sponsor will seek orphan designation 3-6 months prior to planned local submission but then have the designation lapse due to the inability to submit the regulatory application caused by delays in EU or USA approval. This would have the net effect of increasing the TGA workload in having to review another orphan designation application but also lead to delays in Australian patients having access to the medicine due to the ensuing delay in regulatory submission and approval. If the TGA was to proceed with the imposition of a submission deadline, a possible solution to the above problem would be to require sponsors to nominate a target regulatory submission date in their orphan designation application and allow sponsors to update this date through a simple notification procedure.

Comment 2

Should the TGA decide to impose a deadline for sponsors to submit their regulatory application, it may also be appropriate for the TGA to agree to defined review times for sponsor's orphan designation applications. This will provide sponsors with more certainty in the planning of their regulatory applications, which will become of increased importance due to the time limited orphan designation.

General Considerations

Comment 3 – Expedited Pathways

It is noted that the government has committed to the implementation of expedited (priority review and/or provisional approval) pathways for breakthrough drugs. In determining any changes to the orphan drug processes it is of critical importance to patients with rare diseases that orphan drugs will also have access to these expedited pathways.

Comment 4 – Data Exclusivity

One of the key elements to the success of the orphan drugs program in the EU is the provision of an additional incentive to sponsors by way of allocation of additional data protection provisions (10 years for orphan drugs, and 12 years for paediatric applications). It is suggested that the TGA consider adopting a similar approach in Australia in order to support companies like Biomarin who's research and development focus primarily targets rare genetic diseases.